

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
14 October 2004 (14.10.2004)

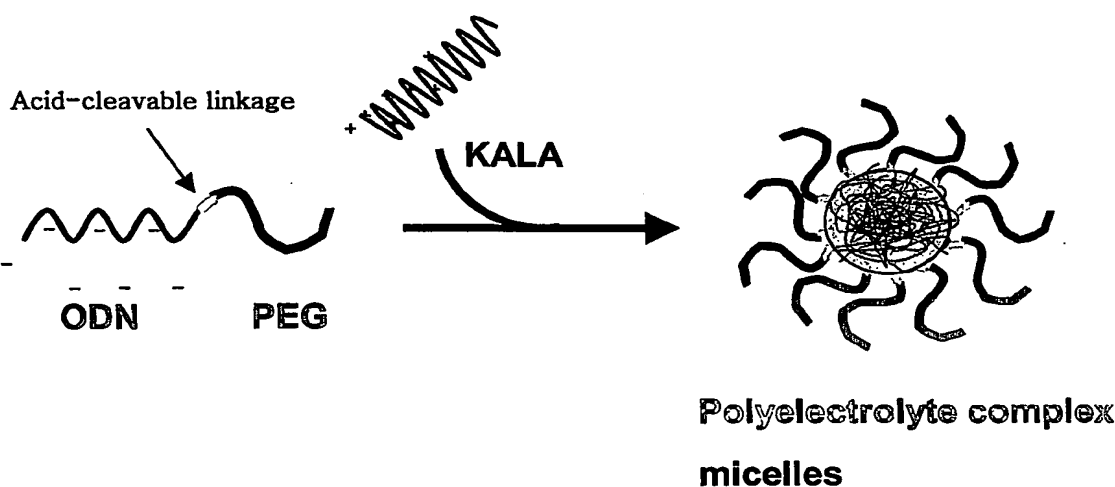
PCT

(10) International Publication Number
WO 2004/087931 A1

- (51) International Patent Classification⁷: **C12N 15/87** (74) Agent: HWANG, E-Nam; 507, New Seoul Bldg., 828-8 Yoksam-dong Kangnam-gu, Seoul 135-080 (KR).
- (21) International Application Number:
PCT/KR2003/000665 (81) Designated States (*national*): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (22) International Filing Date: 3 April 2003 (03.04.2003) (84) Designated States (*regional*): ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).
- (25) Filing Language: English
- (26) Publication Language: English
- (71) Applicant (*for all designated States except US*): KOREA ADVANCED INSTITUTE OF SCIENCE AND TECHNOLOGY [KR/KR]; 373-1, Gusung-dong, Yuseong-gu, Daejeon 305-701 (KR).
- (72) Inventors; and
- (75) Inventors/Applicants (*for US only*): JEONG, Ji Hoon [KR/KR]; 102-401, Hyundai Apt., Daechi-dong, Kangnam-gu, Seoul 135-280 (KR). PARK, Tae Gwan [KR/KR]; 211-1501, Expo Apt., Jeonmin-dong, Yuseong-gu, Daejeon 305-761 (KR).
- Published:
— with international search report

[Continued on next page]

(54) Title: CONJUGATE FOR GENE TRANSFER COMPRISING OLIGONUCLEOTIDE AND HYDROPHILIC POLYMER, POLYELECTROLYTE COMPLEX MICELLES FORMED FROM THE CONJUGATE, AND METHODS FOR PREPARATION THEREOF



(57) Abstract: Disclosed is a conjugate for gene transfer, which is capable of being used for treatment of incurable diseases, comprising an oligonucleotide intended to be transferred into target cells and a hydrophilic polymer, wherein an end of the oligonucleotide is covalently conjugated to the hydrophilic polymer. Also, the present invention discloses polyelectrolyte complex micelles formed from such a conjugate and a cationic polymer or cationic peptide. Such polyelectrolyte complex micelles can effectively transfer oligonucleotides as therapeutic agents into target cells, making it possible to obtain desired activities of the delivered oligonucleotides in target cells even when the micelles are clinically applied at a relatively low concentration. Therefore, the conjugate and the polyelectrolyte complex micelle are very useful in basic life science research and the medical field.



For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.